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Section:	Prescription Drugs	**Effective Date:**	April 1, 2024
Subsection:	Neuromuscular Drugs	**Original Policy Date:**	April 14, 2023
Subject:	Daybue	**Page:**	1 of 4

Last Review Date: March 8, 2024

Daybue

Description

Daybue (trofinetide) oral solution

Background
Daybue (trofinetide) is indicated for the treatment of Rett syndrome. Rett syndrome is a rare genetic neurological disorder that occurs almost exclusively in girls, more rarely in boys, and leads to severe impairments, including their ability to speak, walk, eat, and even breathe easily. The hallmark of Rett syndrome is near constant repetitive hand movements. Rett syndrome is usually recognized in children between 6 and 18 months as they begin to miss developmental milestones or lose abilities they had gained. Rett syndrome is caused by mutations on the X chromosome on a gene called MECP2. The mechanism of action by which Daybue exerts therapeutic effects in patients with Rett syndrome is unknown (1-2).

Regulatory Status
FDA-approved indication: Daybue is indicated for the treatment of Rett syndrome in adults and pediatric patients 2 years of age and older (1).

Daybue contains warnings regarding diarrhea and weight loss (1).

Patients should be advised to stop laxatives before starting Daybue. Interrupt, reduce the dosage, or discontinue Daybue if severe diarrhea occurs, if dehydration is suspected, or if significant weight loss occurs (1).

The safety and effectiveness of Daybue in pediatric patients less than 2 years of age have not been established (1).

Related policies

Policy

This policy statement applies to clinical review performed for pre-service (Prior Approval, Precertification, Advanced Benefit Determination, etc.) and/or post-service claims.

Daybue may be considered **medically necessary** if the conditions indicated below are met.

Daybue may be considered **investigational** for all other indications.

Prior-Approval Requirements

Age 2 years of age or older

Diagnosis

Patient must have the following:

1. Rett syndrome

AND ALL of the following:

a. Documented mutation in the MECP2 gene
b. Prescriber agrees to monitor for diarrhea and significant weight loss

Prior – Approval *Renewal* Requirements

Age 2 years of age or older

Diagnosis

Patient must have the following:

1. Rett syndrome

AND ALL of the following:

a. Patient has had a clinical benefit from therapy (e.g., slowed decline in the severity of signs and symptoms)
b. Prescriber agrees to monitor for diarrhea and significant weight loss

Policy Guidelines

Pre - PA Allowance

None

Prior - Approval Limits

Quantity 24 bottles per 90 days

Duration 12 months

Prior – Approval *Renewal* Limits

Same as above

Rationale

Summary

Daybue (trofinetide) is indicated for the treatment of Rett syndrome, a rare genetic neurological disorder that occurs almost exclusively in girls. Prescribers should monitor patients being treated with Daybue for diarrhea and weight loss. The safety and effectiveness of Daybue in pediatric patients less than 2 years of age have not been established (1).

Prior authorization is required to ensure the safe, clinically appropriate, and cost-effective use of Daybue while maintaining optimal therapeutic outcomes.

References

1. Daybue [package insert]. San Diego, CA: Acadia Pharmaceuticals Inc.; March 2023.
2. About Rett Syndrome: International Rett Syndrome Foundation. 2023. https://www.rettsyndrome.org/about-rett-syndrome/

Policy History

Date	Action
April 2023	Addition to PA
June 2023	Annual review
March 2024	Annual review

Keywords

This policy was approved by the FEP® Pharmacy and Medical Policy Committee on March 8, 2024 and is effective on April 1, 2024.